



# SEQUENCE LISTING

<110> Mitchell, Lloyd G.  
Garcia-Blanco, Mariano A.  
Puttaraju, Madaiah  
Mansfield, Gary S.

<120> METHODS AND COMPOSITIONS FOR USE IN  
SPLICEOSOME MEDIATED RNA TRANS-SPLICING

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<141> 2001-01-08

<150> 09/158,863  
<151> 1998-09-23

<150> 09/133,717  
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<150> 09/087,233  
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<150> 08/766,354  
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 ttcttgca 68

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 tcgagaacat tattataacg ttgc 24

<210> 8  
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| tgcttcaccc gggcctga                    | 18 |
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| cctggacgcg gaagtt                      | 16 |
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| <213> Homo sapien                      |    |
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| ctgggacaag gacactgctt cacccggtta gtagaccaca gccctgaagc c | 51 |
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| <213> Homo sapien  |    |
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| <213> Homo sapien  |    |
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| <210> 18   |    |
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| <212> DNA  |    |
| <213> Homo sapien  |    |
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| <210> 20   |    |
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| <212> DNA  |    |
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| <212> DNA  |    |
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 <223> Oligonucleotide primer complimentary to the  
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       Escherichia coli lacZ gene

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       Escherichia coli lacZ gene  
  
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 <223> Oligonucleotide primer complimentary to the  
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       Escherichia coli lacZ gene  
  
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 <210> 33  
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 <400> 33  
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 <223> Oligonucleotide primer complimentary to the beta

HCG6 gene (accession #X00266)

<400> 34  
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HCG6 gene (accession #X00266)

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HCG6 gene (accession #X00266)

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<223> Oligonucleotide primer complimentary to the  
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Escherichia coli lacZ gene

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Escherichia coli lacZ gene

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<212> DNA  
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<400> 45  
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| <210> 47<br><211> 32<br><212> DNA<br><213> Homo sapien  |    |
| <400> 47<br>gacctcttaa gtagactaac cgattgaata tg   | 32 |
| <210> 48<br><211> 21<br><212> DNA<br><213> Homo sapien  |    |
| <400> 48<br>ctaatagatga tgatgatgat g  | 21 |
| <210> 49<br><211> 21<br><212> DNA<br><213> Homo sapien  |    |
| <400> 49<br>cgcctaataga tgatgatgat g  | 21 |
| <210> 50<br><211> 21<br><212> DNA<br><213> Homo sapien  |    |
| <400> 50<br>cttcttggtgta ctctgtcct g  | 21 |
| <210> 51<br><211> 32<br><212> DNA<br><213> Homo sapien  |    |
| <400> 51<br>gacctctcga gggatttggg gaattatttg ag   | 32 |
| <210> 52<br><211> 21<br><212> DNA<br><213> Homo sapien  |    |
| <400> 52<br>aactagaagg cacagtcgag g   | 21 |
| <210> 53<br><211> 24<br><212> DNA<br><213> Artificial Sequence  |    |
| <220><br><223> trans-spliced product containing Human chorionic gonadotropin gene 6 sequences and Corynebacterium |    |

diphtheriae diphtheria toxin A sequence

<400> 53  
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<210> 54  
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<212> RNA  
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<221> misc\_feature  
<222> (57)...(70)  
<223> Loop comprising a combination of 14 nucleotides  
according to specification

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gcugcag 127

<210> 55  
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<212> RNA  
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according to specification

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gcugcag 127

<210> 56  
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<212> RNA  
<213> Artificial Sequence

<220>  
<223> PTM intramolecular base paired stem

<221> misc\_feature  
<222> (57)...(70)  
<223> Loop comprising a combination of 14 nucleotides  
according to specification

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gcugcag 127

<210> 57  
<211> 132  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> trans-spliced product containing Human chorionic  
gonadotropin gene 6 sequences and Corynebacterium  
diphtheriae diphtheria toxin A sequences

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aaatcttttg tgatggaaaa cttttcttcg taccacggga ctaaacctgg ttatgtagat 120  
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<210> 58  
<211> 18  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Artificial Sequence derived from Escherichia coli  
lacZ gene

<400> 58  
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<210> 59  
<211> 33  
<212> DNA  
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<220>  
<223> Artificial Sequence derived from Escherichia coli  
lacZ gene

<400> 59  
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<210> 60  
<211> 30  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Artificial Sequence derived from Escherichia coli  
lacZ gene

<400> 60  
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<210> 61  
<211> 25  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> trans-spliced product containing Escherichia coli  
lacZ gene sequences and Human chorionic

# gonadotropin gene 6 exon 2 sequences

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<210> 62
<211> 286
<212> DNA
<213> Artificial Sequence

<220>
<223> trans-spliced product containing Escherichia coli
lacZ gene sequences

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agggcggtct cgtctaataa tgggactggg tggatcagtc gctgattaaa tatgatgaaa 180
acgggcaacc cgtggtcggc ttacggcggt gattttggcg atacgccgaa cgatcgccag 240
ttctgtatga acggtctggt ctttgccgac cgcacgccgc atccag 286

<210> 63
<211> 196
<212> DNA
<213> Artificial Sequence

<220>
<223> trans-spliced product containing Escherichia coli
lacZ gene sequences

<400> 63
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agggcggtct gctgttgctg ctgctgagca tgggcgggac atgggcatcc aaggagccac 180
ttcgccacg gtgccg 196

<210> 64
<211> 500
<212> DNA
<213> Artificial Sequence

<220>
<223> trans-spliced product comprising cystic fibrosis
transmembrane regulator-derived sequences and His
tag sequence

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aacgttgctc gagtactaac tggtagctct tctttttttt cctgcagact tcacttctaa 120
tgatgattat gggagaactg gagccttcag agggtaaaat taagcacagt ggaagaattt 180
cattctgttc tcagtttttc tggattatgc ctggcaccat taaagaaaat atcatctttg 240
gtgtttccta tgatgaatat agatacagaa gcgtcatcaa agcatgccaa ctagaagagc 300
atcatcatca tcatcattag gcggccgcca ctgtgctgga tatctgcaga attccaccac 360
actggactag tggatccgag ctccggtacca agcttaagtt taaaccgctg atcagcctcg 420
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ctggaaggtg ccactccac 500

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<211> 20  
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<220>  
<223> Splice junction sequence

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<210> 66  
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<212> PRT  
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<220>  
<223> C terminal residues from glutathione -S-  
transferase

<400> 66  
Asp Tyr Lys Asp Asp Asp Lys  
1 5

<210> 67  
<211> 15  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Artificial sequence comprising sequences derived  
from Escherichia coli lacZ gene

<400> 67  
ggagttgatc ccgtc 15

<210> 68  
<211> 37  
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<213> Artificial Sequence

<220>  
<223> Artificial sequence comprising sequences derived  
from Escherichia coli lacZ gene

<400> 68  
gcagtgtcct tgtgcggtta ccctgcaggg cggcttc 37

<210> 69  
<211> 120  
<212> DNA  
<213> Artificial Sequence

<220>  
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<400> 69  
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tattaactca tttgattcaa aatattttaa atacttcctg tttcatactc tgctatgcac 120

<210> 70  
<211> 24  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Spacer sequence of PTM

<400> 70  
aacattatta taacgttgct cgaa 24

<210> 71  
<211> 47  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Branch point, pyrimidine tract and acceptor splice  
site of PTM

<400> 71  
tactaaactgg tacctcttct tttttttttg atatcctgca gggcggc 47

<210> 72  
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<212> DNA  
<213> Artificial Sequence

<220>  
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<400> 72  
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gatccaccgg 70

<210> 73  
<211> 260  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Binding domain of spacer sequence

<400> 73  
tcaaaaagtt ttcacataat ttcttacctc ttcttgaatt catgctttga tgacgcttct 60  
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ctggaaaact gataacacaa tgaaattctt ccactgtgct taaaaaaacc ctcttgaatt 180  
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aactcattat caaatcacgc 260

<210> 74  
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<220>  
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|  |    |
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| <400> 74<br>cgctggaaaa acgagcttgt tg                           | 22 |
| <210> 75<br><211> 23<br><212> DNA<br><213> Artificial Sequence |    |
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| <400> 75<br>actcagtgtg attccacctt ctc                          | 23 |
| <210> 76<br><211> 36<br><212> DNA<br><213> Artificial Sequence |    |
| <220><br><223> Oligonucleotide                                 |    |
| <400> 76<br>gacctctgca gacttcactt ctaatgatga ttatgg            | 36 |
| <210> 77<br><211> 33<br><212> DNA<br><213> Artificial Sequence |    |
| <220><br><223> Oligonucleotide primer                          |    |
| <400> 77<br>ctaggatccc gttcttttgt tcttcactat taa               | 33 |
| <210> 78<br><211> 33<br><212> DNA<br><213> Artificial Sequence |    |
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| <400> 78<br>ctagggttac cgaagtaaaa ccatacttat tag               | 33 |
| <210> 79<br><211> 35<br><212> DNA<br><213> Artificial Sequence |    |
| <220><br><223> Oligonucleotide primer                          |    |
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 <210> 81  
 <211> 23  
 <212> DNA  
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 <220>  
 <223> Binding domain of PTM molecule  
  
 <400> 81  
 acccatcatt attaggtcat tat 23  
  
 <210> 82  
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 <400> 84  
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 <210> 85  
 <211> 52  
 <212> DNA  
 <213> Artificial Sequence



<220>  
 <223> Random sequence inserted to replace 3' splice site  
  
 <221> misc\_feature  
 <222> (7)...(30)  
 <223> spacer sequence, see SEQ ID NO 70  
  
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 <210> 86  
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 <213> Artificial Sequence  
  
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 <223> Oligonucleotide  
  
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 <223> Oligonucleotide  
  
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 acgccg 66  
  
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 <400> 89  
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<210> 90  
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<220>  
<223> Oligonucleotide

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28

<210> 91  
<211> 30  
<212> DNA  
<213> Artificial Sequence

<220>  
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<400> 91  
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aacataatct tcggcgtcag ttacgacgag taccgctatc gctcgggtgat taaggcctgt 180  
cagttggagg ag 192

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<211> 27  
<212> DNA  
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<220>  
<223> PTM sequences

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27

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<220>  
<223> Oligonucleotide

<400> 94  
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30

<210> 95  
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 <223> 3' splice site  
  
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<220>

<223> Sequence from trans-splicing domain

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47

<210> 101

<211> 1584

<212> DNA

<213> Artificial Sequence

<220>

<223> CFTR PTM

<400> 101

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attaaggcct gtcagttgga ggag 1584
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<210> 102

<211> 323

<212> DNA

<213> Artificial Sequence

<220>

<223> trans-splicing domain of CFTR PTM

<400> 102

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ctgtatctat attcatcatt ggaaacacca atgatatttt ctttaattgg gcctggcata 180
atcctggaaa actgataaca caatgaaatt cttccactgt gcttaatttt accctctgaa 240
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ttaactcatt atcaaatcac gct 323
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<210> 103  
<211> 165  
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<220>  
<223> PTM binding domain

<400> 103  
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<210> 104  
<211> 225  
<212> DNA  
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<220>  
<223> trans-splicing domain of CFTR PTM

<400> 104  
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<210> 105  
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<220>  
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<211> 500

<212> DNA

<213> Artificial Sequence

<220>

<223> reverse complement of trans-spliced product comprising cystic fibrosis transmembrane regulator-derived sequences and His tag sequence

<400> 106

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